Avapritinib Decreased Symptom Burden in Patients With Indolent Systemic Mastocytosis in the Placebo-Controlled PIONEER Study

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Introduction

• Indolent systemic mastocytosis (ISM) is a chronic mast cell disease driven by the KIT D816V mutation
• Patients with ISM may experience life-long debilitating symptoms including the threatening symptoms of systemic involvement (SI) with significant morbidity

Methods

• Retrospective analysis of Part 1 and Part 2 of the PIONEER study
• The study design included a dose-finding (Part 1) followed by a randomized, double-blind, placebo-controlled portion (Part 2) of the PIONEER study
• Patients were randomized to receive avapritinib 25 mg QD (n=10), 50 mg QD (n=10), 100 mg QD (n=10), or placebo (n=9) for 24 weeks, followed by an open-label extension period
• The primary endpoint of Part 1 was the mean change in symptom-related KIT scores from baseline at Week 24 for Part 1 (period 24 weeks)

Results

• Patients experienced an improvement in all ISM symptoms per the ISM Symptom Assessment Form (ISM-SAF) total score
• The mean change in KIT scores was significantly different between the avapritinib groups and the placebo group

Conclusions

• Avapritinib demonstrated a significant and durable improvement in symptoms of ISM
• Long-term safety and efficacy of avapritinib in patients with ISM were observed

References


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